DESIGN OF A LARGE-SCALE CLINICAL TRIAL FOR THE TREATMENT OF SPINAL CORD PAIN USING STEM CELL THERAPY

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EXECUTIVE SUMMARY

Regenerative therapies involving stem cell transplantation are reaching clinical implementation. Large-scale clinical trials are required to determine efficacy, evaluate outcome, and improve techniques. The purpose of this project is to provide an economic assessment of a large-scale clinical trial for a stem cell therapy to take place in Calgary, Alberta, Canada.

The project was conceived as a fourth year design project for engineering students at the University of Calgary. Successful animal studies were previously completed by Mukhida *et al.,* [1] and formed the basis of the clinical scale-up. To the authors' knowledge, this is the only economic evaluation of a large-scale stem cell therapy in the literature.

Current treatment costs of spinal cord injury exceed $650,000 per patient. This project will cost a total of $25.7 million CAD to treat 312 patients over 3 years. Per patient treatment costs will reduce 87% to $81,000 per patient.

This paper details the process economics, expected clinical outcome, bioreactor design, scale-up protocols, and facility design (including bioreactor laboratory in the Heritage Medical Research Building and operating theatre at the Seaman Family MR Research Centre, University of Calgary).

ASSUMPTIONS

The experimental results presented in Mukhida *et al.* [1] were observed in a rat model of spinal cord injury. For this project, we have assumed that clinical results will be similarly effective, so that there is a drive to implement this therapy in clinical neurosurgery. Additionally, we have assumed a few successful clinical cases, and designed a Phase II clinical trial.

PROCESS OVERVIEW

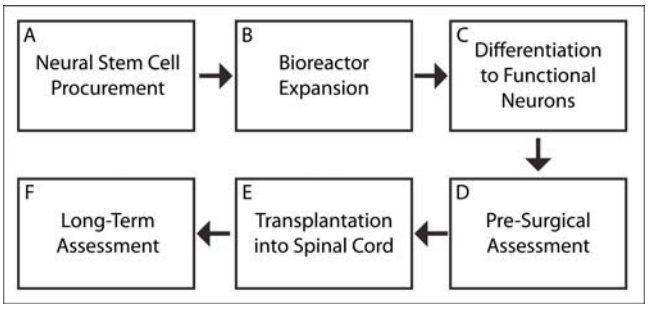

Figure 1: Process overview of neural stem cell therapy for treatment of spinal cord pain. *Following procurement (A), human neural stem cells are expanded in 500 mL computer-controlled bioreactors (B) and differentiated into functional GABAergic neurons (C). Patients are assessed prior to surgical intervention (D), functional GABAergic neurons are transplanted as single cells (E), and patients are carefully monitored to maximize long-term functional recovery (F).*

Table 1: Summary of 3-Year Process Economics

Expenditure	Cost (Million CAD)
Total Capital Investment	$5.468
Annual Operating Costs	$4.253
Annual Surgical Intervention	$1.800
Annual Follow-Up Evaluation	$0.600
TOTAL	**$25.7**

INTRODUCTION

There are over 2.5 million people worldwide suffering from spinal cord injury (SCI) [2]. As of April 2009, there are over 13,500 cases of SCI per year in North America alone [3][4][5], which cost over $9 billion/year to treat [5][6]. Furthermore, over 64% of SCI patients experience neuropathic pain [1], which significantly decreases quality of life. Neuropathic pain is often characterized by sensations of burning,